

METHODS FOR DELIVERING DNA TO MUSCLE CELLS
USING RECOMBINANT ADENO-ASSOCIATED VIRUS VECTORS

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Abstract of the Disclosure

10 The use of recombinant adeno-associated virus (AAV) virions for delivery of DNA molecules to muscle cells and tissue is disclosed. The invention allows for the direct, *in vivo* injection of recombinant AAV virions into muscle tissue, e.g., by intramuscular injection, as well as for the *in vitro* transduction of muscle cells which can subsequently be introduced into a subject for treatment. The invention provides for sustained, high-
15 level expression of the delivered gene and for *in vivo* secretion of the therapeutic protein from transduced muscle cells such that systemic delivery is achieved.

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